(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization

International Bureau



(43) International Publication Date 5 February 2004 (05.02,2004)

PCT

(10) International Publication Number WO 2004/011624 A2

(51) International Patent Classification7:

C12N

19350-9332 (US). SHUEY, David [US/US]; 49 Longview Circle, Berwyn, PA 19312 (US).

(21) International Application Number:

PCT/US2003/024028

(22) International Filing Date:

31 July 2003 (31.07.2003)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

60/399,998

31 July 2002 (31.07.2002) US

(63) Related by continuation (CON) or continuation-in-part (CIP) to earlier application:

US

60/399,998 (CIP)

Filed on

31 July 2002 (31.07.2002)

- (71) Applicant (for all designated States except US): NUCLE-ONICS, INC. [US/US]; 14 Spring Mill Drive, Malvern, PA 19355 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): PACHUK, Catherine, J. [US/US]; 3212 New Park Circle, Lansdale, PA 19446 (US). SATISHCHANDRAN, C. [US/US]; 605 Shepard Drive, Lansdale, PA 19446 (US). CHOPRA, Maninder [IN/US]; 384 Egypt Run Road, Landenberg, PA

(74) Agent: BIEKER-BRADY, Kristina; Clark & Elbing LLP.

101 Federal Street, Boston, MA 02110 (US).

- (81) Designated States (national): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC,
 - LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (regional): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

 without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: DOUBLE STRANDED RNA STRUCTURES AND CONSTRUCTS, AND METHODS FOR GENERATING AND USING THE SAME

(57) Abstract: The present invention relates to novel double stranded RNA (dsRNA) structures and dsRNA expression constructs, methods for generating them, and methods of utilizing them for silencing genes. Desirably, these methods specifically inhibit the expression of one or more target genes in a cell or animal (e.g., a mammal such as a human) without inducing toxicity. These methods can be used to prevent or treat a disease or infection by silencing a gene associated with the disease or infection. The invention also provides methods for identifying nucleic acid sequences that modulate a detectable phenotype, such as the function of a cell, the expression of a gene, or the biological activity of a target polypeptide.

